The Development of Novel Cellular Therapeutics

Some Lessons from the Private Sector

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Cell Genesys Today

Next generation biological therapies for cancer

- Lead program – GVAX® immunotherapy for prostate cancer – in two multinational Phase 3 trials
- Positive Phase 2 data across GVAX® platform – prostate cancer, pancreatic cancer and leukemia
- Complementary clinical stage product platform – CG0070 oncolytic virus therapy for bladder cancer
- Manufacturing capabilities for product launch
- Strong balance sheet

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The History of Cell Genesys

One-slide summary of 17 years!

- 1989 - 1995: The Technology Development years
  - *Gene activation technology for therapeutic proteins*
  - *Transgenic technology for human monoclonal antibodies*
  - *Cell/gene therapy technology for HIV infection*
- 1996 - 1999: The Restructuring years
  - *Spinout of Abgenix (monoclonal antibodies)*
  - *Acquisition of Somatix (GVAX® cellular immunotherapies for cancer)*
- 2000 - present: The Product Development years
  - *4 more acquisitions and 1 more spinout (Ceregene)*
  - *Establishment of large scale manufacturing capabilities*
  - *Advancement of GVAX® Prostate to Phase 3*

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So What Do Biotech and Home Remodeling Have in Common?

Timeline and cost realities

"It takes twice as long and costs twice as much."

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A Specific Example: Amgen's Panitumumab

A timeline experienced by Steve Sherwin

1995: Product selection at Cell Genesys

1996: Initiation of product development at Abgenix

2006: BLA submitted to FDA by Amgen

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Another Specific Example: Genentech's Avastin®

A timeline experienced by Steve Sherwin

1973: Attended lecture by Dr. Judah Folkman at Harvard Medical School

1987: Attended first VEGF Research Project Team Meeting at Genentech

2004: Attended ASCO presentation of successful Phase 3 trial in colorectal cancer

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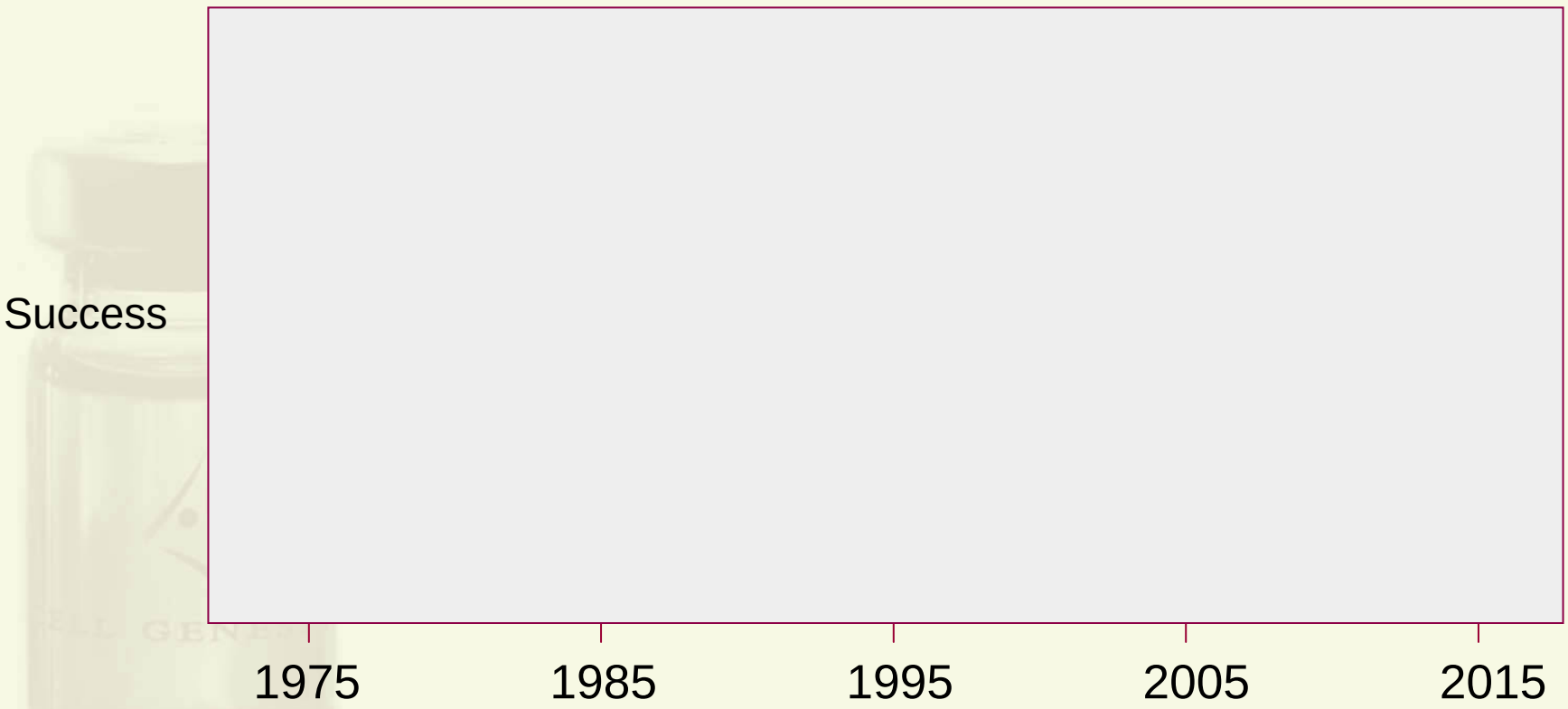
The Twenty Year Lesson!

Novel technology development timelines



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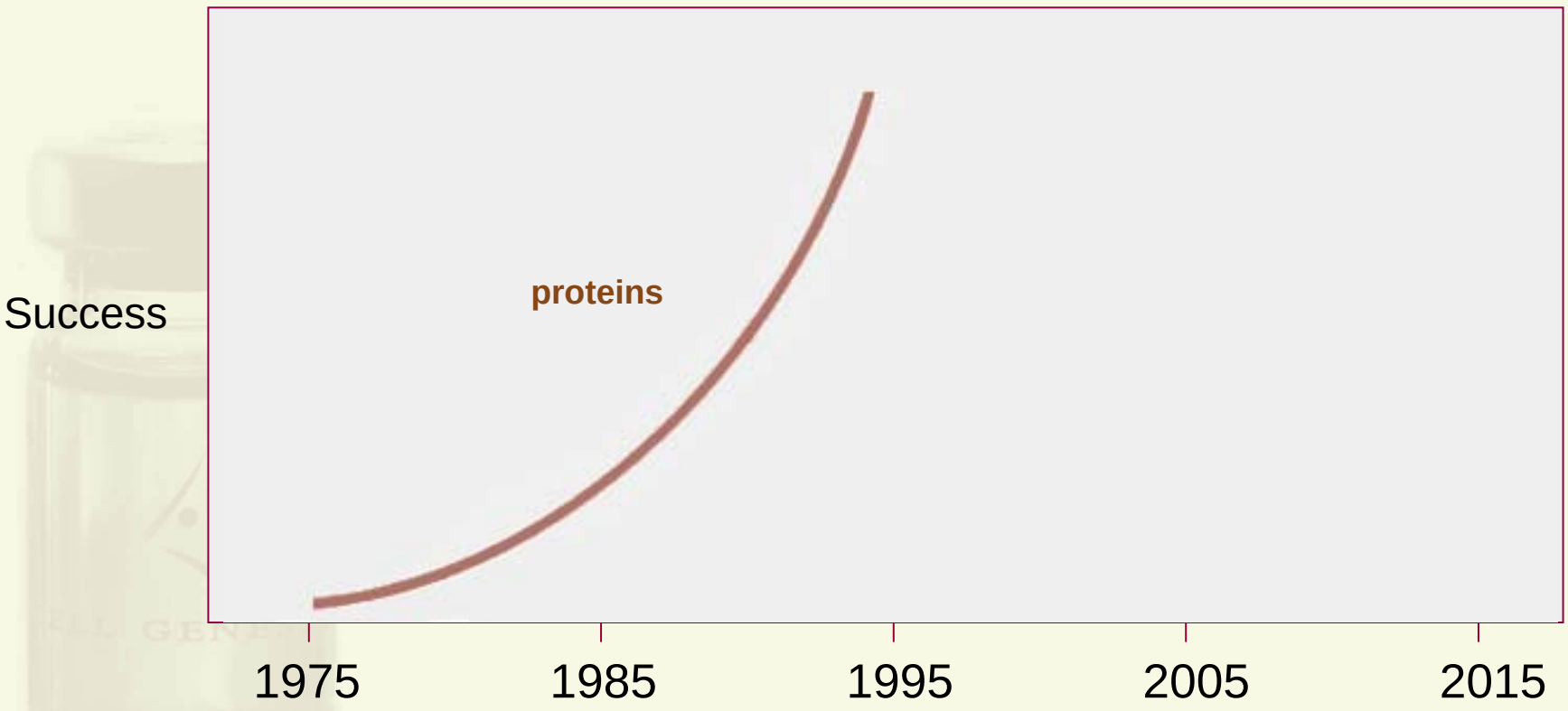

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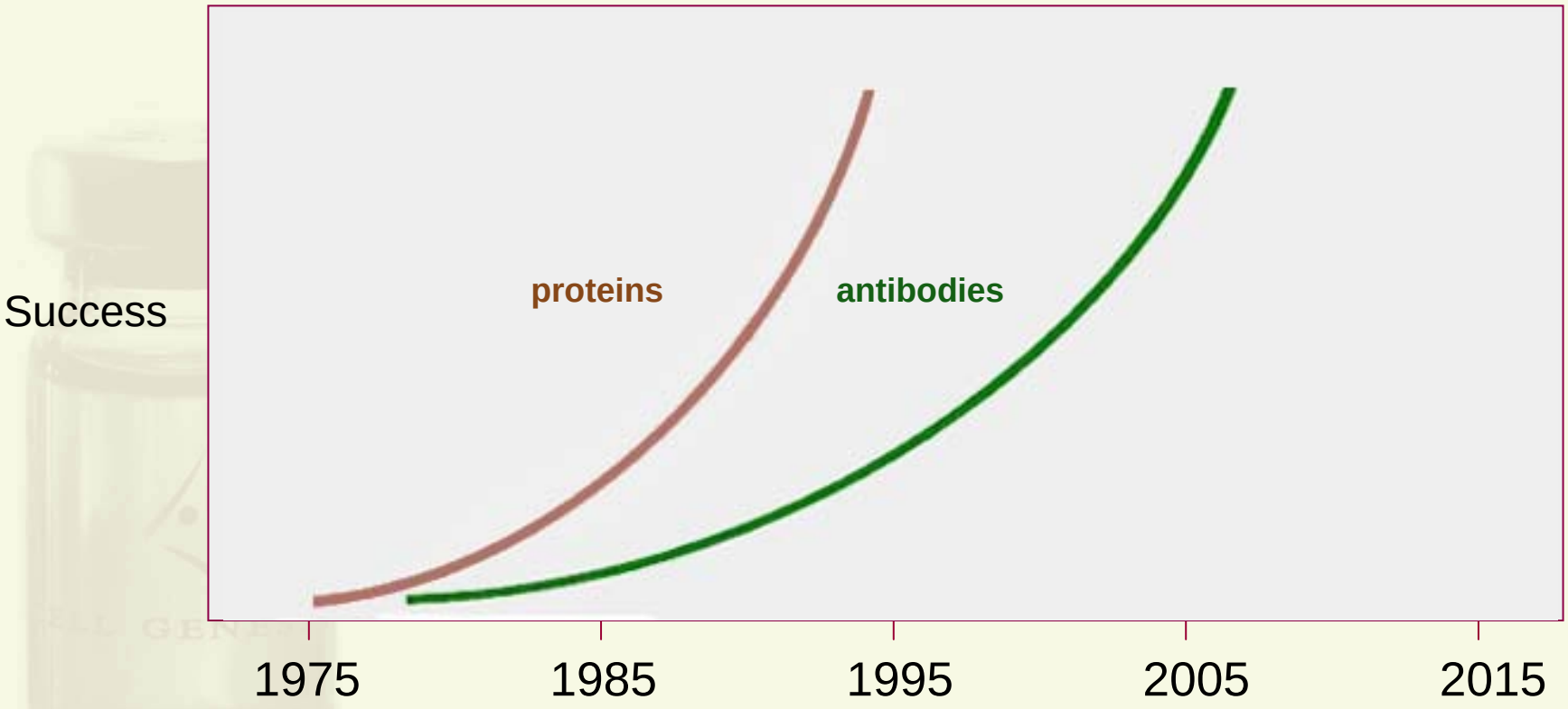

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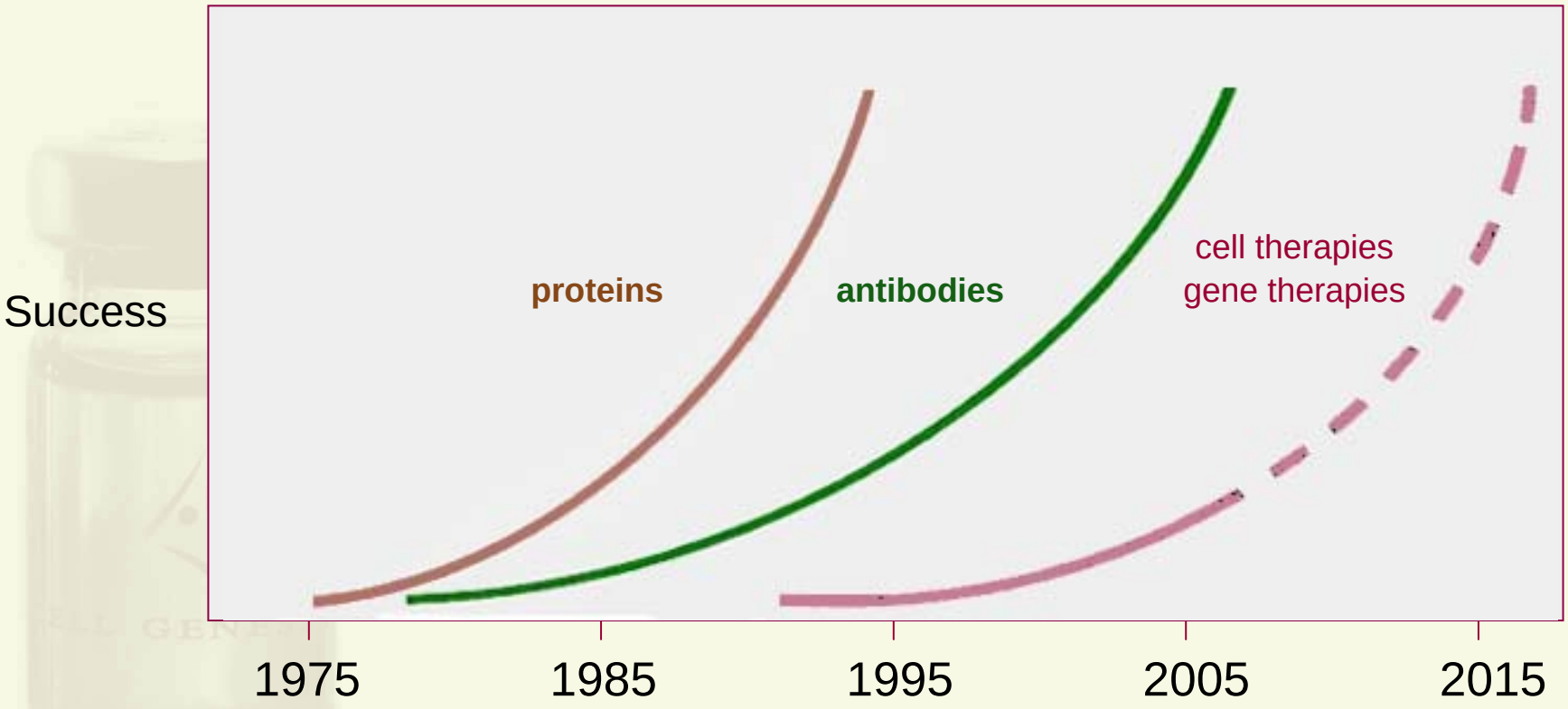

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Why Does It Take So Long?

Multiple challenges

- Technology challenges
- Clinical challenges
- Regulatory challenges

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Technology Challenges

Limitations of first generation technologies

- Therapeutic proteins
  - *Availability of gene expression technology for complex proteins*
- Monoclonal antibodies
  - *Availability of human (humanized) antibodies*
- Gene therapies
  - *Availability of safe and efficient gene delivery (vector) systems*
- Cell therapies
  - *Manufacturing and product characterization at pharmaceutical scale*

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Clinical Challenges

Evaluating new therapies for chronic diseases

- Efficacy measurements in chronic diseases
- Demonstration of long-term safety
- Size, duration and cost of Phase 3 trials

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Regulatory Challenges

Evaluating complex biological therapies

- Adequacy of existing CMC guidelines
- Current regulatory framework for Phase 3
- Implementation of FDA's Critical Path Initiative

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What Are The Potential Solutions?

Some lessons from the private sector

- Plan for longer timelines and more expensive programs (and manage expectations)
- Be willing to turn on a dime
- Dismantle all signs of ego and collaborate

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What Can The Private Sector Do?

Complementary strengths and assets

- What academia does well
  - Basic discovery research
  - Identification of clinical opportunities
  - Identification of clinical limitations
- What the private sector does well
  - GLP pre-clinical studies
  - GMP manufacturing
  - GCP clinical trials

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